BrainStorm Cell Therapeutics to Present at 6th Annual ALS Research Symposia

Presentation on NurOwn Phase 3 data demonstrating evidence of biological effect in ALS; Biomarker data reinforce clinical outcomes

NEW YORK, Nov. 17, 2023 /PRNewswire/ -- BrainStorm Cell Therapeutics Inc. (NASDAQ: BCLI), a leading developer of cellular therapies for neurodegenerative diseases, today announced a podium presentation and panel discussion at the 6th Annual ALS Research Symposium hosted by ALS ONE. The presentation will feature new analyses from the NurOwn placebo-controlled Phase 3 amyotrophic lateral sclerosis (ALS) trial that highlight the biological effect of NurOwn through CSF biomarker data. The presentation, titled, "NurOwn for ALS: Biomarker exploration of NurOwn multimodal mechanism of action on neuroinflammation, neuroprotection and neurodegeneration" will be presented by Bob Dagher, MD, Executive Vice President and Chief Development Officer at BrainStorm.

A copy of the presentation will be available on the Events & Presentation page of Brainstorm's corporate website following the presentation.

"Biomarkers of ALS progression and underlying disease mechanisms are starting to emerge and while the field is still early, there are a number of biomarkers associated with neurodegeneration, neuroinflammation, and neuroprotection that correlate with disease severity" stated Dr. Stacy Lindborg, co-Chief Executive Officer of BrainStorm. "In this analysis, NurOwn demonstrated evidence of a biological effect in data from all Phase 3 trial participants. These data reinforce the clinical outcomes from the trial and provide strong statistical evidence of a NurOwn effect across biomarkers over time."

Phase 3 NurOwn Study Design

The Phase 3 NurOwn trial was a multi-center, placebo-controlled, randomized, double-blind trial designed to evaluate the safety and efficacy of repeat doses of NurOwn in 189 ALS participants. It was conducted at six centers of excellence: University of California Irvine (Dr. Namita Goyal); Cedars-Sinai Medical Center (Dr. Matthew Burford, Dr. Robert Baloh); California Pacific Medical Center (Prof. Robert Miller, Dr. Jonathan Katz); Massachusetts General Hospital (Prof. Merit Cudkowicz, Dr. James Berry); University of Massachusetts Medical School (Prof. Robert Brown) and Mayo Clinic (Prof. Anthony Windebank, Dr. Nathan Staff). Potential participants with ALS were screened during an 18-week run-in period and those who were rapid progressors (defined as participants with at least a 3-point decrease in ALSFRS-R score during the run-in period) were randomized 1:1 to receive three intrathecal injections (8 weeks between each injection) of NurOwn or placebo. Participants were followed for 28 weeks after treatment. The primary endpoints of the trial were safety assessments and a responder analysis of the rate of decline in ALSFRS-R score over 28 weeks, where a response was defined as participants with a 1.25 points/month improvement in the post-treatment versus pre-treatment slope in ALSFRS-R at 28 weeks following the first treatment. Secondary endpoints included the percentage of participants with disease progression halted or improved, ALSFRS-R change from baseline, combined analysis of function and survival, slow vital capacity, tracheostomy-free survival, overall survival, and cerebrospinal fluid biomarker measurements. For more information on the trial, visit https://clinicaltrials.gov/ct2/show/NCT03280056.

About NurOwn®

The NurOwn® technology platform (autologous MSC-NTF cells) represents a promising investigational therapeutic approach to targeting disease pathways important in neurodegenerative disorders. MSC-NTF cells are harvested from each person with ALS and are manufactured using an innovative and proprietary process to secrete neurotrophic factors to target specific neurodegenerative diseases. The lead program for NurOwn is for the treatment of ALS. BrainStorm's long-term commitment to ALS is demonstrated in preclinical research and a series of clinical studies, all of which have been published in peer-reviewed journals.

The NurOwn clinical program has generated valuable insights into the pathology of ALS, as well as disease progression and treatment. Since the initial Phase 3 readout, BrainStorm has shared the full dataset through rigorous peer-reviewed analysis, including: quantification of Floor Effect, which had been noted, but never before explored in depth; evaluation of multiple pre-specified biomarkers, collected at seven different points across 20 weeks during the trial, allowing a longitudinal view; and analysis of genetic data, which represents one of the first ALS trials to prospectively invoke pharmacogenomic analysis of clinical outcome, offering great promise for the development of future treatments for ALS.

About BrainStorm Cell Therapeutics Inc.

BrainStorm Cell Therapeutics Inc. is a leading developer of innovative autologous adult stem cell therapeutics for debilitating neurodegenerative diseases. BrainStorm holds the rights to clinical development and commercialization of the NurOwn® technology platform used to produce autologous MSC-NTF cells through an exclusive, worldwide licensing agreement. Autologous MSC-NTF cells have received Orphan Drug designation status from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) for the treatment of amyotrophic lateral sclerosis (ALS). BrainStorm has completed a Phase 3 trial in ALS (NCT03280056); this trial investigated the safety and efficacy of repeat-administration of autologous MSC-NTF cells and was supported by a grant from the California Institute for Regenerative Medicine (CIRM CLIN2-0989), and another grant from the ALS Association and I AM ALS. BrainStorm completed under an investigational new drug application a Phase 2 open-label multicenter trial (NCT03799718) of autologous MSC-NTF cells in progressive MS and was supported by a grant from the National MS Society (NMSS).

Notice Regarding Forward-Looking Statements

This press release contains "forward-looking statements" that are subject to substantial risks and uncertainties, including the clinical development of NurOwn as a therapy for the treatment of ALS, the future availability of NurOwn to patients, and the future success of BrainStorm. All statements, other than statements of historical fact, contained in this press release are forward-looking statements. Forward-looking statements contained in this press release may be identified by the use of words such as "anticipate," "believe," "contemplate," "could," "estimate," "expect," "intend," "seek," "may," "might," "plan," "potential," "predict," "project," "target," "aim," "should," "will" "would," or the negative of these words or other similar expressions, although not all forward-looking statements contain these words. Forward-looking statements are based on BrainStorm's current expectations and are subject to inherent uncertainties, risks and assumptions that are difficult to predict. These potential risks and uncertainties include, without limitation, management's ability to successfully achieve its goals, BrainStorm's ability to raise additional capital.

BrainStorm's ability to continue as a going concern, prospects for future regulatory approval of NurOwn, whether BrainStorm's future interactions with the FDA will have productive outcomes, and other factors detailed in BrainStorm's annual report on Form 10-K and quarterly reports on Form 10-Q available at http://www.sec.gov. These factors should be considered carefully, and readers should not place undue reliance on BrainStorm's forward-looking statements. The forward-looking statements contained in this press release are based on the beliefs, expectations, and opinions of management as of the date of this press release. We do not assume any obligation to update forward-looking statements to reflect actual results or assumptions if circumstances or management's beliefs, expectations or opinions should change, unless otherwise required by law. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance, or achievements.

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